EDITORIAL

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Patient Advocacy in Research
Merely an Afterthought?

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In 20 years of breast cancer advocacy, I've witnessed major changes in attitude toward advocates on the part of basic researchers, clinical investigators, and others involved with the science of the disease.

Not so long ago, even a seat at the table – much less a vote – was hard to come by. Advocate presence was met with puzzlement, if not outright resistance. How could we possibly understand the complex scientific issues under discussion? What, exactly, was our expertise?

Within the space of a very few years – not coincidentally with the increase in advocate-driven research funding – waves of change began to wash away this resistance. Appreciation for our fundraising efforts, mixed with grudging recognition that people who actually live with a disease have useful perspectives, were contributing factors. In the wake of the 1970s and the women's health movement, medical paternalism was relaxing its grip, and a new generation embraced the patient-doctor relationship as a partnership of equals. In the late 1980s, AIDS activists demonstrated the power of organized pressure on access and policy when lives were at stake. In the early 1990s, the Internet began to connect and empower a steady stream of newly informed patients.

It was in just such an online community, the first breast cancer mailing list, that my own advocacy was born, from a desire to 'pay forward' the information and support offered me when I was diagnosed. What began as a small, personal ripple expanded outwards as I recovered from treatment and moved from helping the members of my support group to reaching out to women in emerging organizations and communities, then to all women with breast cancer.

I joined with other advocates. Not satisfied with offering only emotional support, we called for better, less toxic treatments available to everyone. The standard of care seemed harsh and crude. Some referred to chemotherapy, surgery, and radiation as 'poison, slash, and burn.' Rejecting the metaphors of war, we called for gentler, targeted therapies with less 'collateral damage.' To accomplish this, more and better research was needed.

We were not content with just handing over the research funds. We looked with suspicion at the minor progress made in Nixon's war on cancer[1] and began to raise questions about the direction of cancer research, and the lack of collaboration and support for novel ideas. Funded through the efforts of advocates, programs such as the US Department of Defense Breast Cancer Research Program[1] were born.

In the years since, the presence of advocates has become commonplace in the world of cancer research. We sit on US FDA advisory committees as voting members, on Institutional Review Boards (IRBs), and data safety monitoring boards for clinical trials. We serve on Specialized Programs of Research Excellence (SPOREs) and in the

1 When American President Richard Nixon signed the National Cancer Act into law in 1971, he said, "The time has come in America when the same kind of concentrated effort that split the atom and took man to the moon should be turned toward conquering this dread disease."

Cooperative Groups.[2] We rate grant proposals in study sections, and help determine research priorities. We speak at conferences, and are co-authors of papers, posters, and abstracts. We offer input to industry about trial design and enrolment, drug safety, and access to new therapies in the pipeline. Our activism runs both ways. By translating what we learn of the research back to the patients we serve, we help them to make better, more informed treatment decisions.

In preparing this article, I shared my concerns with Patient Advocates in Research (PAIR), a long-standing mailing list. The flood of responses spoke candidly about our difficulties, as well as our roles and strengths.

Several emphasized the important role we play in encouraging collaboration rather than competition among researchers, helping them to concentrate more on meaningful goals, and less on professional and career concerns. This is no easy task. It is the nature of research to "meander from one disparate place to another," explains Deborah Collyar, President and co-founder of the PAIR mailing list, "which keeps the focus on the individual PIs [Principle Investigators] who compete with each other for grants. It's like a game of musical chairs." To make an impact, she suggests, "We have to speak up, and remind them to act like big boys and girls to get something done."

Colorectal cancer advocate Kate Murphy agrees, "Research can answer interesting but irrelevant questions, provide statistically significant but clinically insignificant results … but in the end, does it matter to patients? Researchers can lose sight of the real goals, and we can keep them honest."

The very presence of advocates can make a difference. "Not only does my face and my story inject reality into the cancer research enterprise, it seems to add some sense of urgency," says Murphy. "We need to get the job done now without quibbling and without egos."

Sometimes, advocates are the only ones who bring a real-world perspective to research discussions, says breast cancer advocate Cheryl Jernigan. "We've got to get beyond incremental steps that simply add to already unsustainable growing healthcare costs, to research that makes a substantial difference in the length and quality of life, that will be accessible in the community setting and not break the bank."

An education in the language and methods of science is critical. Trained advocates challenge biases researchers may have had, by presenting themselves as thoughtful and intelligent peers and colleagues, well versed in the research process. But still they are underutilized at times, and unsure of their roles. "Most researchers who have engaged with us seem to really appreciate our involvement," says Jernigan, "but don't really know when, where, or how to engage us, and we don't always know what it is we can do."

Joyce Graff, advocate for patients with Von Hippel-Lindau (VHL) disease, a genetic blood vessel malformation, has experienced little support from the SPORE on which she serves: "Periodically, they will turn to me in a meeting and ask whether a proposed clinical trial design will appeal to patients." But her opinion is solicited only *after* trial proposals and informed consent documents have been submitted to the IRB.

Being invited to endorse, or recruit for, research studies without participating in their creation is a familiar complaint. I'm not alone in having been invited to join steering committees for registries and clinical trials only after the important initial decisions are made. Substantive roles for advocates can be hard to come by.

But when we are brought in early in the process, we can make a genuine contribution, Collyar feels, "We help them think about how they will actually implement the trial and how to reach as broad an audience as possible, ethnically speaking, as well as eliminating bogus eligibility requirements."

Many of us have experienced last-minute calls and emails from researchers a day or two before

2 Part of the US National Cancer Institute's Translational Research Program, SPORE grants are collaborative, interdisciplinary groups of preclinical and clinical researchers. The NCI Cancer Trials Cooperative Group program involves networks of researchers, cancer centers, and community physicians in the US, Canada, and Europe who jointly conduct large clinical trials following the same protocol.

grant proposals are due. Clearly, the PI has discovered that advocate involvement is required and is scrambling to find someone. We become an obstacle to be surmounted, not a resource to be used.

I've been fortunate enough to have also had the opposite experience. Involved in a major research grant to study brain metastases in breast cancer,[2] my input, and that of other advocates, has been invited and welcomed at all stages of planning and implementation, with the complete confidence and trust of an enlightened PI who has worked with advocates for years.

Central to drug development, regulatory policy is also of intense interest to advocates. I first served as a voting Patient Representative[3] on the FDA's Oncologic Drugs Advisory Committee a decade ago. At the open public hearings, diverse advocacy perspectives are on display, ranging from passionate pleas for access to calls for tightened regulations. While some view the agency as a bureaucratic and unfeeling barrier to access, the FDA's mandate always made sense to me. I too was concerned about risks and benefits of new treatments, about levels of evidence and adequate proof of safety and efficacy. Having witnessed the rise and fall of bone marrow transplants in breast cancer, I knew how catastrophic the premature adoption of unproven therapies could be.

As a graduate of Project LEAD, the National Breast Cancer Coalition's science training course,[4] I fervently believed that advocates needed to understand the research process, and to be able to look beyond immediate needs to what would offer current and future benefit for *all* patients. To that end, I co-developed a free, online training curriculum for the US Cochrane Center in evidence-based healthcare specifically for advocates.[5]

If advocates only understood the research process, I reasoned, they would surely take more nuanced, thoughtful positions on issues of evidence and access. But reason trumps emotion only rarely when lives are at stake, and desperation fuels inflated hopes for treatment efficacy. Described by ethicist Rebecca Dresser as "advocacy's emphasis on the bright side of medical research,"[6] this unwarranted optimism has the potential to increase demand for unproven treatments, and to make it harder to recruit definitive studies. Patient bitterness and public disillusionment with the research enterprise are sure to follow.

Science is hard. "Most of our experiments fail," a researcher I work with reminded me recently. For advocates, removing the blinders of wishful thinking, replacing them with a clear-eyed, cautious view of scientific research, is sobering but necessary. To do so while maintaining a hopeful stance with the patient communities we represent is harder still. But then, no one promised the truth would be easy.

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